



Mission Statement

The goal of the Patient Reported Outcomes (PRO) working group is to identify and recommend a pool of PRO measures appropriate for use as endpoints in NF clinical trials. PROs are any measures of a patient’s health that comes directly from the patient. Examples of PRO measures are questionnaires about patients’ symptoms, functioning, and quality of life.

Group Focus Areas:

The PRO Group has assessed measures in the core domains of general quality of life, disease-specific quality of life, pain and physical functioning using a systematic review and rating method we developed. In addition to updating our previous recommendations as needed, we are now reviewing measures in additional domains related to disfigurement and mental health. Our group also assists other REiNS working groups to identify PRO measures in other areas (such as vision), and we are available to consult with researchers who are considering using PRO measures as NF clinical trial endpoints.

Current Members

| |
|---|
| Vanessa Merker, PhD (Co-Chair) – Veterans Health Administration – Vanessa.merker@va.gov |
| Heather Thompson, PhD (Co-Chair) – California State University, Sacramento - heather.thompson@csus.edu |
| Krizelle Alcantara – Patient Representative |
| Taryn Allen, PhD – Fredrick National Laboratory for Cancer Research |
| Andrea Baldwin, CRNP – National Cancer Institute |
| Carolina Barnett-Tapia, MD PhD – University of Toronto |
| Belinda Barton, PhD - University of Sydney |
| Kim Bischoff – NF Network |
| Ann Blanton, PhD - State University of New York, Cortland |
| Kevin Franck, PhD MBA – Massachusetts Eye and Ear Infirmary |
| Barbara Franklin – Patient Representative |
| Kathy Gardner, MD – Children’s Hospital of Pittsburgh |
| Deborah Gold, MD – Rainbow Babies and Children’s Hospital |
| Jane Grabowski, AuD - California State University, Sacramento |
| Susie Henley, PhD – University College London |
| Cynthia Hingtgen, MD – Michigan State University |
| Kimberley Koetsier, MD – Leiden University Medical Center |
| Staci Martin Peron, PhD – National Cancer Institute |
| Renie Moss – Patient Representative |
| Drea Petersen, MD – Legacy Health |
| Melissa Reider-Demer, NP – University of California Los Angeles |
| Claas Rohl – Patient Representative |
| Tena Rosser, MD – Children’s Hospital Los Angeles |
| Elizabeth Schorry, MD – Cincinnati Children’s Hospital |
| Taylor Smith, PhD – California Polytechnic State University |



| |
|---|
| Heather Thompson, PhD – California State University, Sacramento |
| James Tonsgard, MD – University of Chicago |
| Ana-Maria Vranceanu, PhD – Massachusetts General Hospital |
| Karin Walsh, PsyD – Children’s National Health System |
| Brad Welling, MD, PhD – Massachusetts Eye and Ear Infirmary |
| Pam Wolters, PhD (former chair) – National Cancer Institute |

Endpoints Published (.pdf reference):

- A. Pain Intensity – Numeric Rating Scale-11 (ages ≥8 years) [\[PDF\]](#)
- B. Pain Interference – the Pain Interference Index (ages 6-24 years) and the PROMIS Pain Interference Scale (ages ≥18 years) [\[PDF\]](#)
- C. Physical Functioning – PROMIS Physical Functioning (ages ≥5 years) [\[PDF\]](#)

Endpoints Currently Under Review (Project lead):

- A. General and Disease-Specific Quality of Life (Pam Wolters, PhD and Ana-Maria Vranceanu, PhD)
- B. Hearing Function and Hearing-Related Quality of Life (Heather Thompson, PhD)

Endpoints Being Evaluated (Project lead):

- A. Disfigurement (Vanessa Merker, PhD and Staci Martin, PhD)
- B. Tinnitus and Communication (Heather Thompson, PhD)

Next Major Milestone (Project Lead, Target Date)

The PRO group is reviewing measures related to disfigurement (as it applies to plexiform neurofibromas, cutaneous neurofibromas, facial weakness and other symptoms of NF1 and NF2.) Currently, our focus is in developing a patient-reported rating of disfigurement severity and reviewing existing scales assessing satisfaction with appearance. The PRO Communication Subgroup is beginning their review of measures related to tinnitus.

Meeting Schedule (Coordinator’s contact info):

The REiNS PRO Working Group meets once a month on a rotating schedule to accommodate different members schedules. In 2020, these meetings are in the 3rd week of the month, either on Tuesday 1-2pm ET, Thursday 12-1pm ET, or Monday 4-5pm. All monthly meetings have CART captioning. To be added to our email list, please contact Vanessa Merker at Vanessa.merker@va.gov